PSY44

DAILY ASSESSMENT OF OUTCOMES CAPTURING HOME TREATMENT OF CONGENITAL HEMOPHILIA WITH INHIBITORS (CHWI): DESIGN, DISPOSITION AND IMPLICATIONS OF THE DOSING OBSERVATIONAL STUDY IN HEMOPHILIA (DOSE)

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OBJECTIVES: Acute bleeds in CHwI patients can be managed with infusions of bypassing agents, such as recombinant factor VIIa (rFVIIa). Sequelae of frequent joint bleeding include arthropathy, pain and disability. The majority of bleeds are now treated at home based on physician recommendations or patient/caregiver experience. Consequently, actual treatment patterns and the impact of CHwI on patients and their families are not well understood. This study assessed the feasibility of daily collection of treatment outcomes as reported by CHwI patients and/or their caregivers. **METHODS:** Frequently-bleeding patients with CHwI (average of ≥4 bleeds over a 3-month period) prescribed rFVIIa as first-line therapy were recruited. Each day, patients/caregivers recorded daily activities, QoL (EQ-5D, VAS health/pain), planned work or school for the patient/caregiver, family assessment of anxiety/stress and activity changes via paper diary, and optional electronic interface. When acute bleeds occurred, they recorded symptoms, treatment decisions, and absenteeism/lost productivity. Each medication dose was recorded on an hourly grid including subjective status, pain score, and mixing/administration time. Treatments were verified by comparison of product box-tops to diaries. **RESULTS:** From 52 enrolled patients, 39 patients (75%) completed diaries for a mean of 105.5 days (median:91; range:66–180). Patient participation was facilitated by an average of 13 calls/emails per patient by a dedicated patient support representative. On average, 13.9% of days were bleed days (median:8.2%; range:0–72%). The diary captured 194 bleeding events in 38 patients; 176 treated hemorrhages (158 rFVIIa-treated). Most bleeds were spontaneous (57.4%) and involved joints (69.7%). Thirty-eight patients provided QoL data on 3771 of 3777 eligible days. **CONCLUSIONS:** This study demonstrated the feasibility of daily diary completion by patients/caregivers with CHwI, providing insights into home treatment of CHwI and its impact on patients and their families. High completion rates were facilitated by dedicated patient support and motivated participants.

PSY45

IMPACT OF ACUTE BLEEDING AND ADMINISTRATION OF BYPASSING AGENTS (BPA) ON DAILY ACTIVITIES OF PATIENTS WITH CONGENITAL HEMOPHILIA WITH INHIBITORS (CHWI) AND THEIR CAREGIVERS AND FAMILIES: OBSERVATIONS FROM THE DOSING OBSERVATIONAL STUDY IN HEMOPHILIA (DOSE)

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OBJECTIVES: Patients experience acute bleeding episodes that can be managed with infusions of BPAs. The study aims to assess impact of hemorrhages on patient/caregiver daily activities, absenteeism/productivity, and time spent mixing/administering recombinant factor VIIa (rFVIIa) and plasma-derived activated prothrombin complex concentrates (pd-aPCC). **METHODS:** Frequently bleeding CHwI patients (≥4 bleeds in 3 months) prescribed rFVIIa as first-line treatment, or their caregivers, recorded treatments and daily activity changes for 3–6 months including time mixing/administering treatments. **RESULTS:** For 39 diary patients with 188 initial day of bleeds, 53.7% were reported with activity unchanged, 31.9% slightly changed, 10.6% significantly rearranged, and 3.7% majorly changed for patients/caregivers. For bleed days 3+, 59.6% reported activity unchanged, 17.0% slightly changed, 7.0% significantly changed, and 16.4% majorly changed. On a 10-point scale, median(range) interference was: day 1, 2.0(0–10); day 2, 3.0(0–10); and days 3+, 4.0(0–10). Only 230 of 491 bleeding days were patient work/school days, with 13.5% fully missed, 3.5% partially missed, and 3.9% unproductive. Only 118 of 229 caregiver bleeding days were work/school days, with 9.3% fully missed, 7.6% partially missed, and 0.8% unproductive. Of 176 BPA-treated bleeding episodes, mixing/administration times were reported for 1270 rFVIIa and 80 pd-aPCC injections. Median mixing+administration times were 5.0+5.0 min for rFVIIa and 29.0+24.5 min for pd-aPCC. Both mixing and administration times were significantly shorter for rFVIIa ($p < 0.0001$). The differences were similar for both hemarthroses and muscle bleeds and for on-demand only patients ($P < 0.0001$ for all). **CONCLUSIONS:** Acute bleeding episodes interfere with patient/caregiver/family activities. However, patients disabled by frequent joint bleeds, and non-working caregivers with multiple children with hemophilia may confound simple analysis of work/school interference. An important contributor to the impact of CHwI is burden of treatment; results demonstrate a significant difference per infusion between rFVIIa and pd-aPCC, which parallels the package insert recommendations, although limited patient sample size should be noted.

PSY46

EFFECT OF ACUTE BLEEDING EPISODES ON QUALITY OF LIFE (QOL) IN PATIENTS WITH CONGENITAL HEMOPHILIA WITH INHIBITORS (CHWI) AND THEIR FAMILIES: OBSERVATIONS OF BLEED VS. NON-BLEED DAY QOL FROM THE DOSING OBSERVATIONAL STUDY IN HEMOPHILIA (DOSE) AND IMPLICATIONS FOR ASSESSMENT AND PREDICTION

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OBJECTIVES: QoL assessments in frequently-bleeding CHwI patients and their families are confounded by pre-existing arthropathy and family circumstances. Periodic QoL assessments typically made on non-bleed days, may not provide complete reflections of the burden on patients/families. This study evaluates the impact of bleeding episodes on patients/caregivers/families and the association between monthly QoL scores and patients' average diary experience. **METHODS:** Frequently bleeding CHwI patients (≥4 bleeds in 3 months) or their caregivers, provided daily assessment of EQ-5D (Index, VAS), VAS-pain, and family anxiety/stress/activity change over 3–6 months. Hierarchical models, stratification, and comparison with mean non-bleed scores were used to evaluate instrument responsiveness to bleed/non-bleed days. **RESULTS:** QoL data were recorded by 37 of 39 enrolled patients/caregivers (3771/3777 eligible diary days, 472 bleed/3299 non-bleed days). Median (range) diary duration was 91 (66–180) days, with 8.2% (0–72.2%) bleed days. Median QoL parameters were worse ($P < 0.0001$) on bleed days (Bleed/Non-bleed): EQ-5D index (0.75/0.83), VAS (72.5/85.0), VAS-Pain (4.0/1.0), and for all EQ-5D dimensions. Trends held at a patient level and within hierarchical models ($p < 0.001$). Bleed days had higher ($P < 0.001$) proportions of days with abnormalities in family anxiety/stress (42% vs. 30%) and family activity changes (34 vs. 21%). Few patients/caregivers reported no problems on non-bleed days in EQ-5D Index (13.5%), VAS (2.7%), VAS-Pain (27%), family anxiety/stress (37.8%) and family activity changes (43.2%). For all patients and bleed types, 70.8% of bleed days had >1 QoL score >1 standard deviation (SD) from the patient's mean non-bleed scores, with 30.9% reporting pain scores >1SD from non-bleed baseline. **CONCLUSIONS:** Assessment of CHwI impact on patient/family QoL typically includes periodic (likely non-bleed day) evaluations reflecting baseline abnormalities. However, daily assessment indicated frequent acute bleeds impair QoL beyond patient's non-bleed day baseline. New approaches are required to assess the cumulative impact of acute bleeds during treatment intervals.

PSY47

OBESITY- AND WEIGHT-SPECIFIC HEALTH-RELATED QUALITY OF LIFE INSTRUMENTS FOR OBESE CHILDREN AND ADOLESCENTS

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OBJECTIVES: The prevalence of obesity is increasing not only in adults, but in children and adolescents. Health and psychosocial issues related to obesity have been documented to negatively impact health-related quality of life (HRQoL), which could affect children and adolescents differently than adults. This research reviews the literature on HRQoL instruments utilized in obese children and adolescents. **METHODS:** A systematic search strategy was used to identify published literature between 1997 and December 2009 via PubMed and Ovid databases. The key terms included obesity, quality of life, health-related quality of life, questionnaires, and adolescents. **RESULTS:** A total of 20 studies were retrieved. Fourteen studies were excluded for not including an obesity- or weight-specific questionnaire (n = 12) and assessing the impact of another disease or treatment (n = 2). Five obesity- or weight-specific instruments were identified: Impact of Weight on Quality of Life-Lite (IWQOL-Lite), Impact of Weight on Quality of Life-Kids (IWQOL-Kids), Dutch Eating Behavior Questionnaire (DEBQ), Sizing Me Up, and Sizing Them Up. All of the instruments were developed for children and adolescents, except IWQOL-Lite. Cumulatively, these five instruments measured 17 domains. The most common domains observed in the questionnaires were related to Physical Functioning/Comfort, Emotional Functioning, and Social Life. Other domains measured were Body Esteem, Family Relations, Mealtime Challenges, and School Functioning. The domains that were most impactful in assessing HRQoL were Physical Comfort, Body Esteem, and Social Life. The primary outcome of the studies demonstrated poor HRQoL in obese children and adolescents. **CONCLUSIONS:** Obesity- and weight-specific HRQoL instruments are limited, especially for children and adolescents. With the increasing prevalence of obesity in this subpopulation, there is a significant need for the development of instruments to assess the total impact of this disease. The information provided from these instruments can assist with future obesity treatment in children and adolescents.

PSY48

THE VALUE TO PATIENTS OF TREATING PLAQUE PSORIASIS

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OBJECTIVES: To quantify the value to patients of reducing the severity and size of plaque psoriasis (PsO) skin lesions. **METHODS:** Individuals with a self-reported diagnosis of PsO were recruited from a nationally representative household panel. Individuals completed a web-based choice-format conjoint analysis survey and were